

T1-00007

**Application Number:** T1-00007

**Scientific Score:** 80

**Title:** Human Stem Cell Training at [REDACTED]

*Specific names of individuals and institutions are blacked out to preserve applicant confidentiality where possible.*

### **Proposal Abstract as Submitted by Applicant**

[REDACTED] propose training opportunities in stem cell biology and technology, their application to the treatment of disease, and the legal and ethical issues surrounding the study and use of stem cells. Capitalizing on the new opportunities in human stem cell science, faculty and staff have organized a stem cell center to consolidate our research activities in gene expression, tissue engineering, and the analysis of cord blood stem cells. We have designed a program to support the education and training of fellows in the full spectrum of issues relating to stem cell technology. Scientific coursework will cover renewal and differentiation of embryonic and somatically derived stem cells, the engineering of stem cells and tissues, and the characterization and therapeutic application of stem cells derived from human cord blood. This program will be complemented by teaching from faculty in the humanities, social sciences, and law, who will provide a comprehensive overview of sociological, bioethical and legal issues surrounding stem cell research. The training program will include 16 fellowships; 2 clinical fellows in pediatric hematology/oncology, 6 postdoctoral and 6 predoctoral fellows in molecular biology or bioengineering, 1 predoctoral fellow in humanities, and 1 fellowship in law, to be shared between two students. Science fellows' research will be in one of four major areas; growth, renewal and differentiation of hematopoietic and neural stem cells; engineering synthetic environments that control stem cell self-renewal and fate; gene networks in stem cell differentiation; and stem cells and cancer. Humanities fellows' research will promote the representation of women and minorities in stem cell research or combat health disparities in the development, testing and affordability of stem cell therapeutics. Law fellows will focus their studies on the legal rules, issues and institutions most likely to impact the pursuit and application of stem cell research. This program will develop scholars with varying areas of expertise, but a shared appreciation of the scientific, ethical and legal complexities surrounding this emerging technology.

### **Benefit of this Program to California**

This program will benefit the people and the state of California by providing high-quality training in the scientific, clinical, social, and ethical aspects of stem cell research to the scientists and clinicians who will develop and apply future therapies in this rapidly emerging field.

### **Summary of Review**

This application proposes a type I program that exploits strengths in basic science and clinical training along with the legal and ethical aspects of stem cell biology and regenerative medicine. The institution has received \$1,000,000 for the development of a stem cell center, which has already attracted some key stem cell researchers. Training of clinical fellows will be carried out in collaboration with a children's hospital, which

makes the potential for translation excellent. The applicant pool is superb and the proposed plans for achieving diversity of CIRM trainees are exceptional. The program director is an outstanding scientist who has trained 12 PhD students and 34 post-doctoral fellows, but has no experience in leading a training program like the one proposed. The training faculty also has a very strong record of accomplishment, but few mentors (including the director) have a strong background in embryonic stem cell biology. There is, however, expertise in adult stem cells and in cord-blood related stem cells. Courses in ethics, intellectual property law, and humanities are stellar and feature faculty with a deep knowledge in this area. However, the coursework and program design in stem cell biology was not adequately developed. There was concern among reviewers about the focus of the training program, specifically how training in embryonic stem cells would be incorporated into the program. More description of the exact nature of the core curriculum (e.g., who was teaching which class) and the level of commitment to the training by current faculty would have helped with evaluation of the program. Reviewers recommended a reduction in the number of pre-doctoral and post-doctoral trainees due to the application's inadequate attention to the program design and description of the basic stem cell coursework.

### **Overall Strengths and Weaknesses**

Overall, this is a strong program with regard to the leadership and mentors, ethics and humanities, and emphasis on cord-blood stem cell biology. It also has a large and highly qualified pool of applicants among all trainee levels. However, the proposal presents weaknesses in the specific details of the course design, administration, program management, and depth of knowledge in embryonic stem cell biology.

### **Recommendations**

Highly meritorious and recommended for funding with a reduction of trainee slots. Reviewers encourage the applicant to submit an application at a later date for a supplement to fund these positions when concerns have been addressed.

	Pre	Post	Clinical	Total
Fellows Requested:	8	6	2	16
Fellows Recommended:	6	4	2	12

	Year 1	Total
Budget Requested:	\$ 1,095,184	\$ 3,285,552
Budget Recommended:	\$ 843, 270	\$ 2,529,810